4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-D-0576]

Draft Guidance for Industry: Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of a draft guidance document entitled "Guidance for Industry: Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products" dated July 2013. The draft guidance document provides sponsors of Investigational New Drug Applications (INDs) for cellular therapy (CT) and gene therapy (GT) products (referred to collectively as CGT products) with recommendations to assist in designing early-phase clinical trials of CGT products.

DATES: Although you can comment on any guidance at any time (see 21 CFR 10.115(g)(5)), to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance, submit either electronic or written comments on the draft guidance by November 22, 2013.

ADDRESSES: Submit written requests for single copies of the draft guidance to the Office of Communication, Outreach and Development (HFM-40), Center for Biologics Evaluation and Research (CBER), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448. Send one self-addressed adhesive label to assist the office in processing your requests. The draft guidance may also be obtained by mail by calling CBER at 1-800-835-4709

or 301-827-1800. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

Submit electronic comments on the draft guidance to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Melissa Reisman, Center for Biologics Evaluation and Research (HFM-17), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448, 301-827-6210.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance document entitled "Guidance for Industry: Considerations for the Design of Early-Phase Clinical Trials of Cellular and Gene Therapy Products," dated July 2013. The draft guidance document provides sponsors of INDs for CGT products with recommendations to assist in designing early-phase clinical trials of CGT products. The scope of this guidance is limited to products for which the Office of Cellular, Tissue and Gene Therapies/FDA has regulatory authority. CGT products within the scope of this guidance meet the definition of “biological product” in section 351(i) of the Public Health Service (PHS) Act (42 U.S.C. 262(i)). The guidance does not apply to those human cells, tissues, and cellular-and tissue-based products (HCT/Ps) regulated solely under section 361 of the PHS Act (42 U.S.C. 264), to products regulated as medical devices under the Federal Food, Drug, and Cosmetic Act (the FD&C Act), or to the therapeutic biological products for which the Center for Drug Evaluation and Research (CDER) has regulatory responsibility.

The design of early-phase clinical trials of CGT products often differs from the design of clinical trials for other types of pharmaceutical products. Differences in trial design are necessitated by the distinctive features of these products, and also may reflect previous clinical experience. The draft guidance document describes features of CGT products that influence clinical trial design, including product characteristics, manufacturing considerations and preclinical considerations, and suggests other documents for additional information. Consequently, the draft guidance document provides recommendations with respect to these products as to clinical trial design, including early-phase trial objectives, choosing a study population, using a control group and blinding, dose selection, treatment plans, monitoring and follow-up. Finally, the draft guidance encourages prospective sponsors to meet with FDA review staff regarding their IND submission and offers references for additional guidance on submitting an IND.

The draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent FDA's current thinking on this topic. It does not create or confer any rights for or on any person and does not operate to bind FDA or the public. An alternative approach may be used if such approach satisfies the requirement of the applicable statutes and regulations.

II. Paperwork Reduction Act of 1995

This draft guidance refers to previously approved collections of information found in FDA regulations. These collections of information are subject to review by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (44 U.S.C. 3501-3520). The collections of information in 21 CFR part 312 have been approved under OMB control number 0910-0014.

III. Comments

The draft guidance is being distributed for comment purposes only and is not intended for implementation at this time. Interested persons may submit either electronic comments regarding this document to http://www.regulations.gov or written comments to the Division of Dockets Management (see ADDRESSES). It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at http://www.regulations.gov.

IV. Electronic Access

Persons with access to the Internet may obtain the draft guidance at either http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm or http://www.regulations.gov.

Dated: June 25, 2013.

Leslie Kux,

Assistant Commissioner for Policy.

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